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PRINCIPAL INVESTIGATOR: Andrew S. Bradlyn, Ph.D.

CONTRACTING ORGANIZATION: West Virginia University Research  
Corporation  
Morgantown, West Virginia 26506-6845

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<b>13. ABSTRACT (Maximum 200 Words)</b> The objective of this project is to develop an NF1-specific health-related quality of life (HRQL) instrument for pediatric patients. Semi-structured interviews with children with NF1, their parents, health care professionals, and teachers will yield relevant domains and content that will be used to develop the first-generation instrument. This instrument will then be tested and revised to produce a valid and reliable measure that is sensitive to changes in clinical status. At the conclusion of the first year, we have obtained human subjects approval from both the Army and WVU Institutional Review Boards, and have recruited and completed a number of parent and child interviews. Interviews with health care providers are scheduled and teachers will be scheduled and completed once school has resumed. As the data set is not yet complete and ready for analysis, findings and conclusions are not reported.				
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## 1. INTRODUCTION

The objective of this project is to develop an NF1-specific health-related quality of life (HRQL) instrument for pediatric patients. Semi-structured interviews with children with NF1, their parents, health care professionals, and teachers will yield relevant domains and content that will be used to develop the first-generation instrument. This instrument will then be tested and revised to produce a valid and reliable measure that is sensitive to changes in clinical status. It is our hypothesis that the addition of this disease-specific measure will significantly improve the descriptiveness and quantification of the impact of NF1 and its treatment.

## 2. BODY

The original Statement of Work identified the following tasks in this first year:

- Complete review of literature to gather previously identified HRQL-related topics
- Develop standardized interviews for patients, parents, teachers, and health care professionals
- Pilot interviews and modify as needed
- Conduct interviews of patients, parents, teachers, and health care professionals
- Transcribe interviews, review for functioning areas impacted by NF1.

Additionally, although not stated in the SOW, we were required to gain approval from both the Army/Department of Defense Institutional Review Board for the Protection of Human Subjects and the same body for West Virginia University.

**Status of Work.** Each of the tasks above is again listed, with current status described.

- a. *Complete review of literature to gather previously identified HRQL-related topics.* This task has been completed. As we had noted in our proposal, there have been no prior instruments that specifically assess HRQL for NF1 patients. There are however, several dermatological measures (e.g., SkinDex) which were identified and reviewed for content. Prior literature regarding psychological and psychosocial functioning was reviewed and findings were noted. This information was used to develop the interviews described in the next task. Additionally, this information is being used in the preparation of a manuscript reviewing and discussing the concept of HRQL as it applies to patients with NF1.
- b. *Develop standardized interviews for patients, parents, teachers, and health care professionals.* Each of these semi-structured

interviews has been completed. The interviews are included in the Appendix materials.

- c. *Pilot interviews and modify as needed.* Prior to being used with participants, the interviews were piloted and revised to reflect the feedback received regarding length, complexity, and clarity. Interview revisions are submitted for review and approval by our institutional IRB.
- d. *Conduct interviews of patients, parents, teachers, and health care professionals.* These interviews are currently ongoing. Our initial goal was to have interviews completed by this point, however we have not yet reached that point, primarily due to delays in obtaining the necessary IRB approvals. Although funding for the project began in August 2002, final Department of Defense and West Virginia University IRB approval was not received until March 2003 thereby delaying contact with human subjects. We have scheduled and completed 60% of parent interviews, 25% of child interviews, and 60% of health care professional interviews. Teachers have been identified but not scheduled until the start of the 2003 school year. All interviews have been audiotaped and transcribed for analysis. We have continued actively recruiting subjects through web sites (e.g., Neurofibromatosis Foundation, Neurofibromatosis, Inc.), web bulletin boards, mailings and contacts with NF clinics and state associations, and presentations (e.g., NF, Inc. Board of Directors, family research conference). We anticipate being able to complete the interviews in a timely fashion and to advance to the next steps without further problems.
- e. *Transcribe interviews, review for functioning areas impacted by NF1.* All completed interviews have been transcribed and reviewed for additional content.

### **3. KEY RESEARCH ACCOMPLISHMENTS**

As this stage of the project is still in progress, there have no activities in this category.

### **4. REPORTABLE OUTCOMES**

None as yet. We are preparing a manuscript reviewing and conceptualizing health-related quality of life as it applies to individuals with neurofibromatosis 1, and plan to submit that for peer review and publication in the next reporting period.

### **5. CONCLUSIONS**

The data have not been analyzed at this point, and therefore no conclusions can be drawn.

## **6. REFERENCES**

None

## **7. APPENDICES**

Attached are interview guides for parent, patient, health care professionals, and teachers.

## INTERVIEW GUIDE FOR PARENTS

"Health-Related Quality of Life for Pediatric NF1 Patients"  
HSRRB #A-11540

### Introduction:

*"We are interested in learning more about how having NF has affected your child, (NAME). I'll be asking you questions about your child's health, appearance, and their social and school activities. I'll also ask you questions about how you think your child feels about having NF1. Your observations about how NF has affected (NAME) will be very helpful to us in developing our questionnaires."*

*"Please remember that your participation in this study is voluntary and that any information you give us will be kept as confidential as legally possible. You don't have to answer any questions that you aren't comfortable with or you can stop the interview at any time. I'll be audiotaping the interview, and you can ask to have that stopped if you want to. We keep the tapes in a locked file cabinet in our offices, and they are stored by code number and not by name"*

*"Do you have any questions before we get started?"*

- **"First I'd like to talk about your child's HEALTH."**

- Please tell me about NAME's health right now.
- Is s/he having any particular health problems?
- Has your child had health problems related to NF1 in the past?
- How does your child feel about going to see his/her NF doctor?
- How often does your child see his/her NF doctor?
- Does your child see any other doctors?
  - If so, what types of problems are they treating?
- What medicines is NAME taking now?
- How are your child's senses—by that, I mean how they see, hear, or smell things?

- *Has NF affect how they see, or hear, or their ability to smell things?*
  - *Has having NF1 affected your child's coordination or mobility?*
  - *How is your child's speech?*
    - *Has NF affected how your child talks?*
  - *Has your child had any pain because of their NF?*
    - *Any headaches or any kind of peripheral nerve pain?*
  - *Does your child have any stomach problems or aches and pains?*
  - *How does your child sleep?*
    - *Have there been any sleep problems attributed to NF?*
  - *Has your child had any malignancies or cancer as a result of NF?*
  - *Has your child ever experienced any seizures?*
  - *Has your child been able to do all the regular self-care activities for a child his/her age?*
    - *Eating, dressing, feeding themselves?*
    - *Any trouble eating or swallowing?*
  - *Has your child ever had surgery because of NF?*
  - *How much does NAME seem to know or understand about NF1?*
  - *How does your child feel about having NF1?*
  - *Are there any other health issues that I haven't asked about that you think are important for us to be aware of?*
- **"Now I'd like to ask you some questions about (NAME'S) APPEARANCE."**
  - *Does your child have any physical characteristics such as café-au-lait spots, freckling, or neurofibromas that are usually visible when your child is dressed and engaging in routine activities?*
    - *How noticeable would you say these are?*
    - *Has your child ever commented about these?*
    - *Have they expressed any concern about them?*

- Has your child's growth—either their height or size of their head—been affected in any way by NF?
  - Has your child ever commented about these differences or expressed any concerns about their size?
- Does your child have any other things about their body that are noticeable, such as a sunken chest?
- **"Now I'd like to ask you some questions about (NAME'S) MOOD, BEHAVIOR, AND ACTIVITY LEVEL."**
  - How would you describe your child's mood on most days?
  - Would you say that your child is a worrier, or is frequently nervous or afraid?
  - Would you say that your child is more sad or down than other children his/her age?
  - Can you tell me a little about your child's activity level?
    - Is s/he pretty active or does s/he prefer to engage in quiet activities?
  - Does your child have any behavior problems?
  - How is your child's ability to pay attention and concentrate?
    - Has s/he ever had any trouble being easily distracted or had a hard time staying on task?
  - How cooperative is your child with your requests and the rules in your home?
  - Does your child have any mental health or emotional problems?
  - Are there other important issues regarding your child's mood, behavior, or activity level that we haven't talked about?
- **"Now I'd like to ask you some questions about your child's FRIENDSHIPS AND RELATIONSHIPS WITH FAMILY MEMBERS AND OTHER PEOPLE."**
  - Please tell me a little about your child's friends and friendships. Some children have lots of friends, others only have one or two close friends

*that they may play with, and some children spend most of their time alone. How would you describe your child socially?*

- *Are you satisfied with your child's friendships?*
  - *With their choice of friends?*
- *Are your child's friends aware that s/he has NF?*
  - *How much support do you think your child has from his/her friends?*
- *Has having NF ever caused any problems in your child's friendships?*
  - *Have they had problems with teasing because of their NF?*
  - *Have they been treated unfairly because they have NF?*
- *Now I want to ask about your child's relationships with members of the family. Tell me a little about how the two of you get along.*
- *How does NAME get along with his/her (OTHER PARENT)?*
- *How does your child get along with his/her brothers or sisters?*
- *How does s/he get along with other family members, like aunts and uncles, grandparents and cousins?*
- *Do you have other relatives with NF?*
  - *What has NF been like for them?*
- *How much support do you feel that your child gets from his/her family?*
- *What about your child's relationships with other adults or people in the community, neighbors, teachers, anyone like that?*
- *Are there any other important things that you think we should know about how NF has affected your child's friendships and relationships with family members?*

- **"Now I'd like talk about how your child does in SCHOOL."**
  - *What grade is your child in?*
  - *Tell me a little about your child's grades and how s/he usually does in school.*
  - *Is your child's teacher aware that s/he has NF?*
    - *Does s/he seem to know much about the disorder?*

- *How supportive is your child's teacher?*
- *Does your child have any particular problems with learning?*
- *Has your child ever been tested for school placement because of learning problems?*
- *How is your child's handwriting?*
- *How well does your child read?*
- *Is your child enrolled in any special classes at school?*
  - *Do they have an IEP (Individualized Educational Plan)?*
- *How is your child's school attendance?*
  - *How much school have they missed because of NF?*
- *Are there any other issues related to school and academic performance that I haven't asked about that you think we should be aware of?*

**"We are also interested in interviewing several teachers who have experience working with children with NF1. Are there any teachers you know who have experience working with these children? (This can include teachers who have previously taught or are currently teaching your child)?"**

**"We would like to contact them to interview them regarding their general experiences teaching children with NF1. We're not going to ask them for information specifically about your child, only about their experience teaching children with NF1 in the classroom. We will, of course, keep that information private as well."**

**Teacher's Name?**

**School?**

**City and State?**

**"Are there any other important areas or issues that we haven't talked about?"**

**V2.7**

**July 29, 2003**

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**“Thank you for your help!”**

## INTERVIEW GUIDE FOR CHILDREN

### Health-Related Quality of Life for Pediatric NF1 Patients

HSRRB # A-11540

(Project 1: Interviews)

Introduction: "Thanks for agreeing to talk with us. This will probably take about 30 minutes. We are interested in learning more about how having NF has affected you. I'll be asking a number of questions about your health, school, social activities, and how you feel about having NF. Your answers to these questions are very important to help us understand how having NF affects people's lives. Your participation in this study is voluntary and will be kept as confidential as we can. You don't have to answer any question that you don't want to and can stop this interview without any problem. You can also ask me to stop the tape recorder if you want. We'll keep the tape and the information in a locked file cabinet in our offices. Do you have any questions before we get started?"

1. Health. "First let's talk about your health. Tell me about your health now."
  - A. Are you having any problems now?
  - B. Have you had any problems with your health because of your NF in the past?
  - C. How often do you go to see your NF doctor?
  - D. How do you feel about having NF?
  - E. Do you see any other doctors? If you do, what problems do you see them for?
  - F. How do you feel about having to go to the doctor?
  - G. Are you taking any medications right now?
  - H. How well do you see?
  - I. How well do you hear?
  - J. Any difficulty feeling or touching things?
  - K. Any difficulty with smells?
  - L. Do you have any difficulty walking, or running? Any problems with clumsiness or dizziness?

- M. Has NF affected the way you are able to speak or talk?
- N. Do you have any pain? Any headaches or any pains in your arms or legs?
- O. Tell me about your sleep. Do you have any problems falling asleep? Staying asleep? Or getting up?
- P. Have you ever had any seizures?
- Q. Let's talk about the things kids do everyday. Do you have an difficulty getting dressed? Eating or feeding yourself?
- R. Have you ever had surgery because of NF?

2. Physical Appearance. "Now I want to ask you some questions about how you look."

- A. Do you have anything other people can see like café-au-lait spots or freckling?
- B. Do other people ever comment about those?
- C. Do you have any concerns about how you look?
- D. Do you think your height or weight has been affected in any way by NF?
- E. Do you have any concerns about your size?

3. Psychological functioning. " Now I want to ask your some questions about how you feel and the kinds of things that you do."

- A. How would you usually describe your mood?
- B. Would you say that you are a worrier or are you often nervous?
- C. How often do you feel sad or down?
- D. Tell me a little bit about how active you are. Are you generally pretty active or do you prefer to do quiet activities?
- E. Do you have a hard time sitting still?
- F. Are there things that you do at home or school that get you into trouble?

G. How good are you on following your parent's directions and the rules at your house?

4. Social functioning. "Now I want to ask you some questions about your friends and how you get along with other people."

- A. Some kids have a lot of friends, others have only one or two close friends that they play with and some kids spend most of their time alone. How would you describe yourself?
- B. Are you happy with the number of friends that you have and how you get along with them?
- C. Do your friends know that you have NF?
- D. What do they think about you having NF?
- E. How much help or support do you think you get from your friends?
- F. How do you get along with your mother?
- G. How do you get along with your father?
- H. How do you get along with your brothers and sisters?
- I. What about with other members of your family, like aunts and uncles, grandparents and cousins? How do you get along with them?
- J. Do you have any relatives with NF?
- K. What has NF been like for them?
- L. How much help or support do you think you get from your family?
- M. What about getting along with other people? Like neighbors or teachers?

5. School and cognitive functioning. "Let's talk now about how school is going."

- A. What grade are you in?

- B. Tell me what kinds of grades you usually get?
- C. Is your teacher aware that you have NF?
- D. Do your teachers seem to know much about NF?
- E. How understanding is your teacher?
- F. Do you have any particular problems in school?
- G. Do you have any trouble paying attention or staying on task, getting your work done?
- H. How would you say your handwriting is?
- I. How well do you read?
- J. Do you get any special help in school?
- K. Are you in any special classes at school?
- L. Have you missed much school because of NF?

## INTERVIEW GUIDE FOR TEACHERS

### Health-Related Quality of Life for Pediatric NF1 Patients

HSRRB # A-11540

(Project 1: Interviews)

Introduction: "Thank you for agreeing to speak with us. As you know, we are interested in learning more about the effects of NF on children. As part of that process, we are talking with a number of teachers to learn more about how children with NF perform in the classroom and your observations in this regard will be very helpful. I won't be asking you about any specific child, just your general observations about students with NF. Your participation in this study is voluntary and will be kept as confidential as legally possible. You may decline to answer any question that you do not feel comfortable with, may ask to have the audio-tape recording stopped, and/or may terminate this interview at any time without penalty. The tape and transcribed interview will be stored in a locked filecabinet in the investigator's office. Audiotapes will be labeled with a 4-digit code number assigned to each participant to maintain anonymity."

- A. What is your age range? 20 – 30 years   31 – 40 years   41 – 50 years   over 51 years
- B. Gender?      Male      Female
- C. How many years of teaching experience do you have? \_\_\_\_\_
- D. How many years have you taught NF1 students? \_\_\_\_\_
- E. How many NF1 students have you taught? \_\_\_\_\_
- F. In what classes or grades have you had students with NF? \_\_\_\_\_
- G. Can you tell me a little about what you know about NF?
- H. I want to ask you to think about the students with NF you've taught - in general, what can you tell me about how they have done in school?
- I. Did you notice particular subjects they did best in?
- J. Did you notice subjects that are particularly difficult?

- K. Compared to others in the class, how are grades usually?
- L. Do you know if any were tested for placement or to detect any specific learning disabilities or problems?
- M. Did they usually have an IEP or special education plan?
- N. How about speech? Do you ever have any difficulty understanding what the students were trying to say?
- O. How about reading ability?
- P. What about handwriting? How would you describe these students' handwriting?
- Q. Did they seem to have any problems with motor coordination that you have noticed?  
(Get description if problem is reported.)
- R. Did they seem to have any difficulty in visual-spatial organization? (Get description.)
- S. Let talk a little about social relations. How do the other children respond to these students?
- T. To your knowledge are the other children aware that these students have NF?
- U. Do these students seem to have friends within the classroom?
- V. Are you aware of any teasing or problems with and the other children?
- W. What about appearance? Do they have any noticeable signs of having NF?
- X. How did they seem to feel about this?
- Y. How about their ability to pay attention in class?
- Z. Do they seem more distractible or get off task more easily than your other students? Did you notice anything about these students' mood or emotions in school?
- AA. How about behavior in the classroom?

BB. Did they have any difficulty staying in their seat or following the rules?

CC. Have you ever had reason to speak with the parents or the school counselor because you were concerned about mood or behavior?

DD. Is there anything else you can tell me about how NF has affected the students?

Thank you.

## INTERVIEW GUIDE FOR HEALTH PROFESSIONALS

### Health-Related Quality of Life for Pediatric NF1 Patients

HSRRB # A-11540

(Project 1: Interviews)

Introduction: "As you know, we are in the process of developing a quality of life instrument for use in children with NF. As a part of that process, we are interviewing medical professionals who have experience treating NF patients to gain a better understanding of the types of problems that children with NF face. Your participation in this study is voluntary and will be kept as confidential as legally possible. You may decline to answer any question that you do not feel comfortable with, may ask to have the audio-tape recording stopped, and/or may terminate this interview at any time without penalty. The tape and transcribed interview will be stored in a locked filecabinet in the investigator's office. Audiotapes will be labeled with a 4-digit code number assigned to each participant to maintain anonymity."

- A. What is your age range? 20 – 30 years    31 – 40 years    41 – 50 years    above 51 years
- B. Gender?    Male    Female
- C. What is your medical/nursing specialty?
- D. How many years have you been in practice since your residency/since your graduation?
- E. Could you tell me a little about your NF clinic?
- F. How many NF patients you follow?
- G. How often do patients typically come to clinic?
- H. What would you say are the most common medical problems in NF child and adolescent patients?
- I. What types of treatments are used with these problems?
- J. How successful are these treatments?
- K. Can you tell me approximately what percentage of your patients have needed surgery and give some examples for the types of surgery that have been required?

L. Do you use the Riccardi scale to describe impairment in your patients? If so, where do most of your patients fall on that scale?

M. Are there medications that you might routinely prescribe for NF patients? Can you tell me what those are and give me a rough estimate about what percentage of your patients might require that type of medicine?

N. What are some of the common cosmetic and/or dermatologic problems that you see in your child and adolescent patients?

O. What are some of the common vascular abnormalities that you have found in your patients?

P. What are some of the common neurologic problems experienced by your patients?

Q. Approximately what percentage of your patients experience those problems?

R. I understand adolescence can be a time when NF problems become exacerbated. What types of problems do you usually see during this period?

S. What are some of the common learning problems or educational problems your child and adolescent patients might have?

T. Are you aware of any behavioral or mood difficulties that your child patients have?

U. Are you aware of any school of learning problems that your NF patients have?

V. Do parents often talk with you about their child's behavioral or school problems?

W. In your experience, how does having a child with NF generally affect the parents?

X. Are there any problems experienced by NF patients we haven't discussed so far?